

**Genzyme Corporation**

500 Kendall Street  
Cambridge, MA 02142  
Tel 800-745-4447

January 21, 2011

RE: U.S. Supply of Fabrazyme® (agalsidase beta) for February – April 2011

Dear Healthcare Provider,

We are writing to provide you with new information regarding the U.S. supply of Fabrazyme.

Supply for February, March, and April 2011

- Patients currently receiving Fabrazyme who normally get infusions every other week are eligible to receive one 1mg per kilogram dose per month for February, March, and April 2011.
  - For patients who normally have an infusion schedule other than every two weeks, please consult a Genzyme case manager for information about dosing.
  - We will be able to provide 5mg vials of Fabrazyme to support flexible dosing.
- We will provide specific details regarding shipments beyond April 2011 before the beginning of May.

Availability of Fabrazyme for patients not currently receiving Fabrazyme

We are also pleased to announce that, **starting in February 2011, a very limited amount of Fabrazyme will be available to treat people with Fabry disease who are not currently receiving Fabrazyme.** New (or re-start) patients will receive Fabrazyme at the same allocation that current patients receive.

**Providing Fabrazyme treatment to a small number of new patients will not change the amount of Fabrazyme current patients can receive.** The current allocation is the greatest number of doses per patient that Genzyme can currently provide. Increasing the allocation to all current patients by an additional 1 mg/kg dose would require much more Fabrazyme than we will need to add a small number of new patients.

Genzyme has worked closely with the Fabrazyme Stakeholders Working Group (FSWG) to make the decision to allow some new patients to start Fabrazyme treatment. This is a difficult decision that takes into consideration the ongoing constraint on Fabrazyme supply and the needs of people with Fabry disease who have not had access to enzyme replacement therapy since the Fabrazyme shortage began.

The FSWG has prepared guidance to help healthcare providers determine if they should request Fabrazyme for their untreated patients. Please refer to the enclosed [guidance document](#). Because Fabry disease is a complex condition, there is no clear way to decide which patients are in greatest need of treatment. The FSWG guidance provides general guidelines to the medical community and are not requirements for starting a patient on Fabrazyme treatment. Genzyme and the FSWG have determined that **Genzyme will fill requests from healthcare providers to start new patients in the order that requests are received.**

Requests will be filled for the small number of patients who can be accommodated without impacting the current treatment allocation for current patients. If and when that limit is reached, Genzyme will maintain a waiting list of requests for Fabrazyme treatment and will continue to fill requests in the order they were received as more supply becomes available.

**Requests will be accepted starting Wednesday, February 9, 2011 at 12 p.m. E.S.T.** Only a healthcare provider may request treatment for a patient not currently receiving Fabrazyme. To do so, please visit [www.Fabrazyme.com/request](http://www.Fabrazyme.com/request). Once a request is processed Genzyme will contact the person submitting the request to confirm the request and indicate whether there is sufficient supply to start the patient on Fabrazyme or the patient has been placed on the waiting list.

#### General Information

The information in this letter is based on our current best estimate of Fabrazyme supply. Increasing the availability of Fabrazyme remains our highest priority. At this time, we are still working with very limited inventory, so even minor changes to our current manufacturing plan can impact availability of Fabrazyme. We will continue to do our best to inform you of any shipping delays that might affect you or your patient's infusion schedule.

For support regarding insurance and billing issues, infusion agency questions, or additional information about the supply of Fabrazyme, or to give Genzyme your feedback, healthcare providers and patients should contact their Genzyme Case Manager at 1 (800) 745-4447, Option 3 or Medical Information at 1 (800) 745-4447, Option 2.

Sincerely,



Daniel Gruskin, MD  
Senior Director, US Medical Affairs

**Guidance to the U.S. Fabry Community:  
Requesting Fabrazyme® (agalsidase beta) for currently untreated  
patients during the period of supply constraint**

***Prepared by the U.S. Fabrazyme Stakeholders Working Group\****

**January 21, 2011**

Fabry disease is a complex condition and there is no clear way to decide which patients are in greatest need of treatment. The criteria below are meant as general guidelines to the medical community to help healthcare providers determine if they should request Fabrazyme for their untreated patients while supply constraints persist.

- Patients with any of the following clinical features are a higher priority for initiation of Fabrazyme:
  - GFR 25 - 90 ml/min/1.73m<sup>2</sup>
  - Urine protein > 300 mg/day or >300 mg/g creatinine
    - Control of proteinuria with ACE inhibition or ARBs is highly recommended
  - LVH or diastolic dysfunction without heart failure
  - Significant pain not controlled by symptomatic therapy
  - Significant gastrointestinal symptoms not controlled by symptomatic therapy
- Patients with none of these manifestations are a lower priority for initiation of Fabrazyme.

\*The Fabrazyme Stakeholders Working Group (FSWG) includes representatives of the Fabry Support and Information Group (FSIG), the National Fabry Disease Foundation (NFDF), the Fabry Registry North American Board of Advisors, and Genzyme Corporation. Please note that some individuals who participated in the FSWG are employees of Genzyme and other individuals or their institutions or organizations receive or have received funding from Genzyme for research, educational activities, and other purposes.



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January 21, 2011

RE: Important Update on U.S. Supply of Fabrazyme® (agalsidase beta) for February – April 2011

Dear Fabry Community,

We are writing to provide you with new information regarding Genzyme's supply of Fabrazyme for February, March and April 2011.

If you are currently receiving treatment with Fabrazyme and, prior to the Fabrazyme shortage, you normally received Fabrazyme infusions every other week; we will be able to supply enough Fabrazyme for you to receive **one dose (1 mg per kilogram) per month for February, March, and April 2011.**

- If you received infusions more or less frequently than every two weeks, please contact your Genzyme Case Manager or your doctor's office for information about your dosing for February through April 2011.
- We will be able to provide 5mg vials of Fabrazyme to support flexible dosing during this time.

Also, starting in February 2011, a limited amount of Fabrazyme will be available to treat people with Fabry disease who are not currently receiving treatment. Genzyme has worked closely with the Fabrazyme Stakeholders Working Group to make the decision to allow some new patients to start Fabrazyme treatment. In making this decision, we considered the ongoing constraint on Fabrazyme supply and the needs of people with Fabry disease who have not had access to enzyme replacement therapy since the Fabrazyme shortage began.

**Providing Fabrazyme treatment to a small number of new patients will not change the amount of Fabrazyme current patients can receive.** The current allocation is the greatest number of doses per patient that Genzyme can currently provide. Increasing the allocation to all current patients by an additional 1 mg/kg dose would require much more Fabrazyme than we will need to add a small number of new patients.

Physicians may submit a request to Genzyme starting on Wednesday, February 9, 2011 for patients who wish to start receiving treatment with Fabrazyme. These patients will receive Fabrazyme at the same allocation that current patients receive, for instance, a patient who would normally be prescribed 1 mg per kilogram body weight every two weeks would be eligible to receive one 1 mg per kilogram dose each month in February through April.

**Requests will be filled in the order they are received for the small number of patients who can be accommodated without impacting the current treatment allocation for patients currently on Fabrazyme.** If and when that limit is reached, Genzyme will maintain a waiting list of requests for Fabrazyme treatment and will continue to fill requests in the order they were received as more supply becomes available.

While the information in this letter is based on our current best estimate of Fabrazyme supply, it is Genzyme's highest priority to increase the availability of Fabrazyme. We are still working with very limited inventory, so even minor changes to our current manufacturing plan can impact availability of Fabrazyme. As such, please always confirm your infusion appointment with your doctor or infusion site a day or two before your appointment. We will continue to do our best to inform you of any shipping delays that might affect your infusion schedule.

We will contact you again before the beginning of May to provide you with an update regarding shipments of Fabrazyme beyond April 2011. Please do not hesitate to call your Genzyme Case Manager at 1 (800) 745-4447, Option 3 if you have any questions regarding this update or any feedback for Genzyme.

Sincerely,

Pamela di Cenzo, Vice President  
Patient & Product Services



**Genzyme Corporation**  
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Cambridge, MA 02142  
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January 21, 2011

RE: Important Update on U.S. Supply of Fabrazyme® (agalsidase beta)

Dear Fabry Community,

We are pleased to announce that, starting in February 2011, a limited amount of Fabrazyme will be available to treat people with Fabry disease who are not currently receiving treatment.

Genzyme has worked closely with the Fabrazyme Stakeholders Working Group to make the decision to allow some new patients to start Fabrazyme treatment. In making this decision, we considered the ongoing constraint on Fabrazyme supply and the needs of people with Fabry disease who have not had access to enzyme replacement therapy since the Fabrazyme shortage began.

If you would like to begin treatment with Fabrazyme, please speak with your physician to discuss your treatment options and the key considerations outlined below.

- Physicians will be able to submit a request to Genzyme starting on Wednesday, February 9, 2011 for patients who wish to start treatment with Fabrazyme.. The date that a patient starts treatment with Fabrazyme will depend on administrative considerations and availability of Fabrazyme.
- **Requests will be filled in the order they are received** for the small number of patients who can be accommodated without impacting the current treatment allocation for patients currently receiving Fabrazyme. If and when that limit is reached, Genzyme will maintain a waiting list of requests for Fabrazyme treatment and will continue to fill requests in the order they were received as more supply becomes available.
- Please note that patients who want to start treatment with Fabrazyme will receive Fabrazyme based on the supply allocation in place for the Fabry community at the time of their treatment begins.
  - **Current Fabrazyme Allocation:** Patients who are normally prescribed 1 mg per kilogram body weight every two weeks are eligible to receive **one dose (1 mg per kilogram) per month in February, March and April 2011.**
  - Example: A patient who begins Fabrazyme treatment in March and would normally be prescribed 1 mg per kilogram body weight every two weeks would be eligible to receive one dose (1 mg per kilogram) per month in March and April 2011.

**Providing Fabrazyme treatment to a small number of new patients will not change the amount of Fabrazyme current patients can receive.** The current allocation is the greatest number of doses per patient that Genzyme can currently provide. Increasing the allocation to all current patients by an additional 1 mg/kg dose would require much more Fabrazyme than we will need to add a small number of new patients.

Increasing the availability of Fabrazyme remains our highest priority. While the information in this letter is based on our current best estimate of Fabrazyme supply, we are still working with very limited inventory, so even minor changes to our current manufacturing plan can impact availability of Fabrazyme.

If you have any questions or need help understanding your insurance coverage for starting treatment with Fabrazyme, please call your Genzyme Case Manager at 1 (800) 745-4447, Option 3.

Sincerely,

Pamela di Cenzo, Vice President  
Patient & Product Services